CAPITOL STREET

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Platform Technology FDA Guidance Overdue

MRNA, CRISPR, Cell & Gene, CDMOs Likely Benefit

Relevant Companies

Our Take & Next Up

The Food and Drug Administration (FDA) is due to release platform technology guidance that may be vague in nature at first but more specific/helpful upon finalization 2H24. FDA continues to suffer from a lack of highly qualified staff. The agency is also encountering innovative therapies that have unique regulatory barriers including CRISPR and mRNA therapies (non-COVID applications). To address their limitations, the agency is leaning on the private sector to assist in easing regulatory challenges and identifying applications. A potential beneficiary of the new designation is the rare & ultra rare disease space which may see more therapeutics being developed and succeed from the utility of novel techs that allow multiple different diseases to be addressed through the "plug and play" of different targets.

Key Points

CRISPR and mRNA platforms are expected to be early beneficiaries of the pathway. CRISPR-based and mRNA-based tools are being widely used to explore different ways of editing genetic expression and present potential therapies for areas of unmet need including rare diseases. Developers in the CRISPR & mRNA space include BEAM, EDIT, NTLA, CRSP, MRNA, ARCT, CVAC, and BNTX, among others. The sickle cell disease gene therapy, Casgevy (CRSP & VRTX), is the first FDA-approved therapy utilizing CRISPR/Cas9. Future approvals using CRSP's approved platform could leverage Casgevy data to accelerate the FDA review process. While safety concerns remain with off-target impacts resulting from CRISPR editing, there is regulatory optimism around mitigating risk with AI and other novel tools.

Contract development and manufacturing organizations (CDMOs) are likely to weigh in on platform tech guidance, as the designation also applies to processes. CDMOs have become a part of the regulatory conversation for novel therapies as they are relied upon by startups and novel companies. Currently, CDMOs lack formal ways to engage with the FDA, despite increasing usage by drug developers to shorten development time and their knowledge of novel drug manufacturing processes and bottlenecks. Platform technology designation offers the opportunity for CDMOs to engage with the FDA and have their IPs evaluated, which reduces the regulatory barriers for therapies that use their products.

Cell and gene therapies continue to garner federal attention from a policy & regulatory perspective. At the end of 2023, Senator Cassidy (R-LA) requested info from stakeholders on improving gene therapy access, particularly for those with ultra-rare diseases. The request solicited information on gene therapy pricing, contract design, plan management of financial risk, supply chain intermediaries, and the current level of provider access.

BACKGROUND

What is a platform technology?

- The pathway formed under the Consolidated Appropriations Act passed in December 2022 (which also contained FDA user fee reauthorizations) here. The draft guidance was legislatively required to be published no later than one year after the date of enactment, so the agency is way overdue.
- Platform technologies have the potential to be incorporated in or used by more than one drug or biological product. Sponsors may also "reference or rely upon data and information" from a previous application for a drug or biological product that incorporates or uses the same platform technology—if the data is submitted by the same sponsor with permission from the sponsor.

Draft guidance is not likely to be detailed, disappointing novel tech developers. The pathway may be product specific as the agency seeks to strictly define what qualifies as platform technology. Guidance is also likely to discuss program intent, and the level of documentation that should be submitted to obtain platform technology approval. However, we believe that the guidance is unlikely to provide the level of granularity that developers are looking for including the specific number on the number of products that are needed to be eligible for designation according to FDA CBER Regulatory Counsel, Phillip Kurs, comments at CASSS's CMC Strategy Forum.

Currently, one of the formal pathways for potential platform tech to interact with the FDA is through drug master files (DMFs). DMFs provide confidential, detailed information about facilities, processes, or articles used in the manufacturing, processing, packaging, and storing of drug products. The FDA is allowed to review the technical content of DMFs in connection with the review of applications that reference them. This allows drug developers to reference DMF materials while protecting the intellectual property of the DMF holder.

We expect the draft guidance to be released ASAP with final guidance likely 2H 2024 (election season).

- Following draft guidance release, we expect a comment period with final guidance at about the same time as the Presidential election.
- We expect an innovation-friendly backdrop regardless of election outcome, particularly since the pathway enjoys bipartisan lawmaker support. Trump leads Biden in swing states and could potentially re-take the White House this November.

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